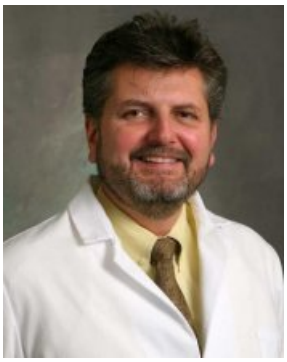
CIRM Funded Clinical Trials

A Phase 1b Safety Study for MRI guided delivery of AAV2-GDNF for the treatment of Parkinson's disease

Disease Area:	Parkinson's Disease
Investigator:	Krystof Bankiewicz
Institution:	Brain Neurotherapy Bio
CIRM Grant:	CLIN2-11661 (Pre-Active)
Award Value:	$5,510,462
Trial Sponsor:	Brain Neurotherapy Bio
Trial Stage:	Phase 1
Trial Status:	Launching
Targeted Enrollment:	N/A



Krystof Bankiewicz

Details:
Parkinson's Disease is a neurodegenerative disease that is thought to be the outcome of the gradual breakdown of dopaminergic neurons in the brain, which are a type of cell that produces a chemical in ones' brain known as dopamine. This decrease in the brain dopamine content can result in symptoms such as uncontrollable shaking of the hands, slowed movement, rigid muscles, loss of automatic movements, speech changes, bladder problems, constipation, and sleep disorders. In the US alone, approximately 60,000 people are diagnosed with PD each year and it is expected that almost one million people will be living with the disease by 2020.

This trial is using a gene therapy approach to promote the production of a protein called GDNF, which is best known for its ability to protect dopaminergic neurons, the kind of cell damaged by Parkinson's Disease. The approach seeks to increase dopamine production in the brain, alleviating PD symptoms and potentially slowing down the disease progress.

Design:
This is a Phase 1b trial.

Goal:
To evaluate safety and preliminary efficacy.

Source URL: https://www.cirm.ca.gov/clinical-trial/phase-1b-safety-study-mri-guided-delivery-aav2-gdnf-treatment-parkinson%E2%80%99s-disease